
A universally applicable skin sheet for Dystrophic Epidermolysis Bullosa via next-generation gene editing, iPS cell technology and tissue engineering

Grant Award Details

A universally applicable skin sheet for Dystrophic Epidermolysis Bullosa via next-generation gene editing, iPS cell technology and tissue engineering

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-12590

Project Objective: To develop an autologous, gene corrected iPSC-derived skin sheet for treating RDEB patients with any mutation(s) in the COL7A1 gene

Investigator:

Name:	Marius Wernig
Institution:	Stanford University
Type:	PI

Disease Focus: Epidermolysis Bullosa, Skin Disease

Human Stem Cell Use: iPS Cell

Award Value: \$1,229,040

Status: Pre-Active

Grant Application Details

Application Title: A universally applicable skin sheet for Dystrophic Epidermolysis Bullosa via next-generation gene editing, iPS cell technology and tissue engineering

Public Abstract:**Research Objective**

We will develop a cell therapy for a rare skin disease. Patient-derived iPS cells will be genetically corrected and differentiated into epithelial sheets to be grafted on skin wounds.

Impact

In this proposal we will develop a universal genetic correction strategy for all COL7A1 which will be a prerequisite for the commercial viability of our iPS cell-based cell therapy.

Major Proposed Activities

- Replacing a medium size fragment of the COLLAGEN7A1 locus
- Excision of the entire COLLAGEN7A1 locus
- Replacement of the entire COLLAGEN7A1 locus with a normal copy
- Differentiate corrected iPS cells into skin cells
- Develop a clinical-grade cell purification system for skin cells
- Verify that manufactured skin cells are functional.

Statement of Benefit to California:

Our ultimate goal is to bring our iPSC-based therapy into the clinic. Our product will have to be commercially viable to ensure sufficient funding through Phase III clinical testing, FDA approval, and production at scale to provide it to the entire patient community. This therapy will directly benefit DEB patients in California. More Californians will benefit from future therapies based on our platform. Finally, academic and commercial development will benefit California's economy.

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